

Attorney Docket No.: **PENN-0882**  
Inventors: **Dreyfuss and Wang**  
Serial No.: **10/532,203**  
Filing Date: **January 9, 2006**  
Page 3

**REMARKS**

Claims 1-8 are pending in this application. Claims 4-8 have been withdrawn from consideration. Claims 1-3 have been rejected. Claims 2-8 have been canceled. Claim 1 has been amended. No new matter has been added by this amendment to the claims. Applicants are respectfully requesting reconsideration of in view of the amendments to the claims and the following remarks.

**I. Election/Restriction**

Applicants acknowledge that the Restriction Requirement has been deemed proper and made Final and that the Examiner has withdrawn the species election. Accordingly, the Examiner has opened examination to compounds that replace or enhance the function of SMN to alleviate or reduce phenotype of cells with low SMN protein levels. Applicants also acknowledge that the Examiner has stated that the method of treating a neurodegenerative disease or disorder with low SMN protein levels comprising administered compounds of Formula I, II and III are stated to be free of the art. Applicants have canceled claims 4-8 without prejudice, reserving the right to file continuing applications on the canceled subject matter.

**II. Rejection of Claims Under 35 U.S.C. 112, Second Paragraph**

Claims 2 and 3 have been rejected under 35 U.S.C. 112, second paragraph, as being indefinite for failing to particularly point out and distinctly claim the subject matter which applicant regards as the invention. The Examiner suggests that the claims are indefinite and vague in claiming a compound comprising formulas I, II or III as one of skill could not ascertain the

Attorney Docket No.: **PENN-0882**  
Inventors: **Dreyfuss and Wang**  
Serial No.: **10/532,203**  
Filing Date: **January 9, 2006**  
Page 4

metes and bounds of the claims where a compound contains another compound. Claims 2 and 3 were also rejected under 35 U.S.C. 112, second paragraph, as being particularly vague and indefinite since applicant is claiming compounds 1-39 comprising formulas I, II and III yet does not disclose the particular structures of the compounds in the claims.

Applicants have canceled claims 2 and 3 making the rejection as it pertains to those claims moot. Withdrawal of this rejection is respectfully requested.

### **III. Rejection of Claims Under 35 U.S.C. 112, First Paragraph**

Claims 1-3 have been rejected under 35 U.S.C., 112, first paragraph, because the specification does not enable any person skilled in the art to which it pertains, or with which it is most nearly connected, to use the invention commensurate in scope with the claims. The Examiner suggests that while the specification is enabling for certain compounds of Formula I, II and III for the treatment of spinal muscular atrophy, it does not reasonably provide enablement for every single compound that replaces or enhances function of SMN or the treatment of all neurodegenerative diseases or disorders with low SMN protein levels. Applicants respectfully traverse this rejection.

At the outset, Applicants have canceled claims 2 and 3 making the rejection moot as it pertains to those claims. Further, in an earnest effort to advance the prosecution and facilitate allowance of the claims, Applicants have amended claim 1 to recite that the method of the invention involves treating a neurodegenerative disease or disorder that is spinal muscular atrophy and further wherein the compound used is selected from

Attorney Docket No.: **PENN-0882**  
Inventors: **Dreyfuss and Wang**  
Serial No.: **10/532,203**  
Filing Date: **January 9, 2006**  
Page 5

the group consisting of Compound 11, Compound 12, Compound 13, Compound 14, Compound 15, Compound 16, Compound 17, Compound 18, Compound 19, or Compound 20. Support for this amendment can be found throughout the specification as filed, but in particular at pages 10-11 where the formulas of Compounds 11 through 20 are taught, at pages 17-19 where it is taught that the compounds as claimed have activity to increase low SMN levels, and also pages 1-4 where it is taught that spinal muscular atrophy is associated with low SMN levels. Accordingly, the claim as amended meets the requirements of 35 U.S.C. 112, first paragraph, and withdrawal of this rejection is respectfully requested.

#### **IV. Rejection of Claims Under 35 U.S.C. 102(b)**

Claim 1 has been rejected under 35 U.S.C. 102(b) as being anticipated by Chang *et al.* (2001). The Examiner suggests that Chang *et al.* (2001) disclose that proximal spinal muscle atrophy is a disease characterized by degeneration of the spinal cord and that survival of motor neuron genes is typical but loss of function of both copies of one gene, SMN1 is correlated with development of the disease. Further, the Examiner suggests that this reference teaches that the severity of disease is correlated with the amount of intact SMN protein, as well as teaching that administration of sodium butyrate ameliorated symptoms of the disease in transgenic mice. Applicants respectfully traverse this rejection.

As discussed *supra*, Applicants have amended claim 1 to recite that the method of the invention involves treating a neurodegenerative disease or disorder that is spinal muscular atrophy and further wherein the compound used is selected from

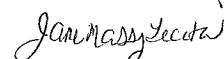
Attorney Docket No.: **PENN-0882**  
Inventors: **Dreyfuss and Wang**  
Serial No.: **10/532,203**  
Filing Date: **January 9, 2006**  
Page 6

the group consisting of Compound 11, Compound 12, Compound 13, Compound 14, Compound 15, Compound 16, Compound 17, Compound 18, Compound 19, and Compound 20. Chang *et al.* (2001) disclose the treatment of spinal muscular atrophy by increasing the level of SMN protein through use of compound, sodium butyrate, to shift the alternative splicing pattern of SMN2-derived pre-mRNA. Nowhere does the paper teach or suggest use of the compounds as claimed in amended claim 1. MPEP 2131 states that in order to anticipate an invention the cited reference must teach each and every limitation of the claim. Accordingly, Chang *et al.* (2001) cannot anticipate the invention of claim 1 as amended. Withdrawal of this rejection is respectfully requested.

**V. Conclusion**

Applicants believe that the foregoing comprises a full and complete response to the Office Action of record. Accordingly, favorable reconsideration and subsequent allowance of the pending claims is earnestly solicited.

Respectfully submitted,



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